NORD®
National Organization for Rare Disorders

ANNUAL REPORT 2015

Alone we are rare. Together we are strong.®

NORD Mission Statement

The National Organization for Rare Disorders is a unique federation of individuals, voluntary health agencies and other health related organizations dedicated to helping people with rare "orphan diseases." NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and services.

Alone we are rare. Together we are strong.®

Advocacy • Education • Patient Services • Research

©2016 The National Organization for Rare Disorders.
The NORD name and tagline are registered trademarks of the National Organization for Rare Disorders.

Dear NORD Members and Friends:

At the National Organization for Rare Disorders, our primary focus is to help people and families affected by rare diseases. We are doing everything we can to speed up progress to find cures, empower rare disease patient organizations, and develop resources that educate patients, families, and doctors.

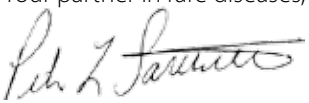
We are intensely aware of the struggles – emotional, financial, and more – that families face throughout their battles with rare diseases. Because of support from our donors, NORD helped more than 4,000 people access life-improving medical care and treatment in 2015 that they could not otherwise afford. Our annual events, Rare Disease Day® and NORD's Rare Diseases & Orphan Products Breakthrough Summit, brought all stakeholders together to raise awareness, make connections, and identify new areas for progress.

As I reflect on this past year and look ahead to 2016, I am struck by how NORD is driving progress for all 7,000 rare diseases and the 30 million people they affect. Our Policy team was vital in advocating for legislation that has the potential to help millions of children and families. We funded research grants and developed more than 20 new natural history studies. We launched an award-winning redesign of our website, which has made it easier for people to find and share our online resources. Because we know that no single organization can tackle these issues alone, we grew our Membership program to more than 250 patient member organizations. In September, we were pleased when ***"The Week"*** magazine named NORD as its ***"Charity of the Week,"*** recognizing our value and trustworthiness.

NORD's work would not be possible without the generosity of our funders and donors, Membership Organizations, Corporate Council partners, and public contributions. Working together, our supporters have donated critical funds and shared their stories to help us toward cures for all types of rare diseases.

At NORD, we set out to do great things. I am pleased to share some of our recent accomplishments in this report.

Your partner in rare diseases,



Peter L. Saltonstall
President and CEO

PATIENT ASSISTANCE

Parents Thank NORD for Saving Daughter's Life

Laurie and Chuck Eallonardo were thrilled at the birth of their twins, Jenna and Caden. Laurie felt happy and relieved that she had been able to carry them for 38 weeks, almost to full term, which can be harder with twins.

One afternoon, during a normal feeding time with 5-month-old Jenna, their baby girl suddenly shrugged her shoulders and rolled her eyes. "It wasn't a big jolt, and could have easily been overlooked, but my gut told me something wasn't right," said Laurie.

Over time, Jenna's erratic movements occurred more often. She began having episodes of bending forward and clenching her body repeatedly. "We were desperate to figure out what was wrong. Finally, an EEG confirmed she was having seizures. We had no idea at that time that using typical seizure medicines would have no effect on Jenna. We also did not realize how neurologically devastating her type of seizures could be," said Laurie and Chuck.

After months wasted on an ineffective treatment, doctors at the Children's Hospital of Los Angeles diagnosed her with Infantile Spasms, a catastrophic age-specific epilepsy syndrome that has its onset within the first 12 months of life, with most cases appearing between 3 and 7 months of age. Jenna was experiencing more than 100 seizures per day. This number is common with the condition, according to NORD's Physician Guide to Infantile Spasms, written with Cristina Y. Go, MD, and O. Carter Snead III, MD, FAAN, and Jenna's parents remember being horrified when they learned this number because it meant that some of Jenna's leg twitches, which had seemed like normal behavior for a baby, were actually part of her seizures.

After receiving the diagnosis, Jenna was prescribed the only approved treatment available at the time, adrenocorticotropic hormone (ACTH). The cost was partly covered by

NORD through its Patient Assistance Programs. **"NORD got the ACTH medication to us immediately while our medical group needed more time to authorize and figure it out. We didn't have an extra minute to waste,"** Laurie said.

"We knew within five days it worked," Laurie recalls. The seizures stopped and Jenna had her first smile since they had begun.

"The ACTH treatments were ultimately a miracle cure for Jenna. After four long months of severe and frequent seizures, Jenna became seizure free [and] to this day our family celebrates the anniversary of Jenna's 'Happy Day,'" said Laurie.

Jenna's neurologist, Dr. Pantea Sharifi Hannauer of UCLA & Pediatric Minds Early Childhood Treatment Center, told the family that she has never seen an infantile spasms patient turn out so well.

Laurie and Chuck recently made a donation to NORD, a 501(c)(3) charity, as a thank you. Laurie and her family are very grateful to NORD for providing medication to them, total strangers.

"Jenna's story [is] really is beyond belief. I want the staff, volunteers, and supporters of NORD to know how critical and life-altering your organization is," Laurie said.

"Your organization helped save our daughter's life when she was a baby. Jenna had severe Infantile Spasms (catastrophic seizures) for four months. NORD paid for the ACTH medication – the injections stopped her seizures."

Making an Impact

74 programs are available to assist patients and their families

4,039 people receive NORD's financial and medication assistance through those programs

180 calls per day into NORD's call center

"Every one of us preparing for medical careers will see patients with rare diseases, and the extent to which we prepare ourselves for this reality will determine the impact we can have on these patients' lives."

– Sophia Walker,
Medical Student and NORD Volunteer

"Hearing from NORD's patient speakers has enabled us, at Northeastern University, to learn about the patient experience from Day 1. Learning about rare diseases from patients and caregivers has provided our future health care professionals with new knowledge that will help them become more compassionate and dedicated providers."

– Nicole Curtis
Student, School of Pharmacy, Northeastern University

EDUCATIONAL INITIATIVES

Better Care.
Understanding that it still takes too many years and too many doctor visits for people with rare disease to receive an accurate diagnosis, NORD intensified its educational efforts this year. More than 40,000 healthcare professionals viewed print or digital editions of our Neurological Rare Disease Special Report, co-published with Frontline Medical Communications. In a follow-up survey, 90% of 142 respondents said it would help them screen patients more proactively and treat them more effectively.

A Brighter Future.
In February, we traveled to the nation's largest gathering of medical students, the annual meeting of the American Medical Student Association (AMSA), where we established the first-ever rare disease booth. We hosted patients and caregivers from NORD's Member Organizations for a "Meet the Patients" session and created a scavenger hunt for medical students to bring rare diseases to life beyond the traditional blurb in a textbook. NORD also established its first-ever free student membership and launched a special newsletter for students preparing for healthcare careers.

Creating Connections.
We hosted 44 patient meetings across the U.S. where we connected more than 1,000 patients with 50 medical experts knowledgeable about specific rare diseases. Through these meetings, patients and caregivers who might never have met another person with their same disease are able to share experiences and hope.

More Awareness.
We redesigned our website in 2015 to provide additional information and resources to patients, families and rare disease stakeholders. Since launching the new site, our traffic has grown to 1 million sessions per month! The website offers a variety of resources, including our unique databases for finding specific rare disease information and patient organizations. Learn more by visiting rarediseases.org.

Making an Impact

Over 1,300 Rare Disease Reports are available on NORD's website

We reach over 1.2 million medical professionals and students through partnerships with medical communications channels

Visitors from **230+ countries** come to NORD's website for information and resources

"The fellow patients and/or caregivers you meet will be family to you."

– Jill Ziegler, patient meeting attendee

"One of the only places that ever knows of my disease (CIDP). Thank you NORD."

– Jade Diquattro, on Facebook

"Thank you NORD for all the good you do."

– Jade Diquattro, on Facebook

ADVOCACY

National Progress.
Since 1983's passing of the Orphan Drug Act, NORD has ensured that the rare disease perspective is at the table when important policy and regulatory decisions are made and 2015 was a banner year for progress

21st Century Cures.
In May, the House Energy & Commerce Committee approved proposed legislation called the 21st Century Cures Initiative, one of our nation's largest efforts to spur the development of new medicines. NORD worked closely with committee staff to assure that the bill included provisions to support the rare disease community, and we will work closely with Senate as it develops its own version of the legislation.

Helping Kids.
More than half of the 30 million Americans with rare diseases are children. We are proud to have led the charge to renew the Rare Pediatric Disease Priority Review Voucher (PRV) Program with one of NORD's most successful advocacy sign-on campaigns to-date, with 115 supporting organizations.

STATE REPORT CARD

Making an Impact

FDA

1/3 of all new drugs approved are to help rare diseases

www

Conducted 10 webinars for NORD members and the general rare disease community

Submitted written testimony on Legislation in 30 states

ADVOCACY

Ensuring Access to Clinical Trials.
In October, the Ensuring Access to Clinical Trials Act of 2015 was signed into law by the President of the United States. We are honored to have worked with our Members Organizations, the Cystic Fibrosis Foundation and the Muscular Dystrophy Association, to advocate for its passage. Now, people who receive Social Security Income and Medicaid will not lose government medical benefits because of the compensation they receive from participating in much-needed clinical trial research to find new treatments.

A Roadmap for State Improvement.
In October, NORD released the first-ever State Policy Progress Report to track health care policies that affect the rare disease community on a state-by-state basis. The report is now being used by multiple state coalitions to highlight policies in need of improvement.

Making an Impact

Hosted over 100 meetings with state legislators on key policy initiatives

5,000 Sign-Ups to join the RareAction Network℠

Traveled over 5,000 Miles to meet with advocates and elected officials during our RareAction Road Tour.

"How to handle the copayments for my cancer treatment was extremely stressful, but because of the assistance you provided, I can forget that stress. Thank you for providing some peace."

Jerry, Advanced Renal Cell Carcinoma

ACCESS ASSISTANCE

Serving the Community

Since 1987, NORD's patient assistance programs (PAP's) have supported patients and families impacted by rare disease across the country to obtain life-saving or life-sustaining treatment that they would not otherwise be able to afford. Our dedicated team fields on average 180 calls per day. Representatives from NORD are available over the phone for enrolled patients, serving as the patients point of contact for accessing their medication.

"Thanks for helping me reach my dream to meet my doctor. This visit cleared so many doubts that I had about my condition with Gaucher. I will always be thankful." –Frances, Gaucher Disease

Improving Access to Life-Improving Treatment and Care

During 2015, NORD hosted a Patient Access and Reimbursement Seminar for industry representatives to lead conversations on the legal, operational and financial issues affecting patients' access to needed therapies. This special event was open for companies developing treatments for patients with rare diseases, and NORD lead the conversation on PAP's in relation to the current legal, operational, and reimbursement issues affecting patients' access to needed therapies.

Making an Impact

74 programs are available to assist patients and their families

4,039 people receive NORD's financial and medication assistance through those programs

180 calls per day into NORD's call center

RESEARCH

Research Support

NORD's long-running research grant program, funded mostly by patients, patient organizations, and donations provides seed money to researchers. In many cases, our grants are the only sources of funding for the study of specific diseases and we are proud to say that NORD's research grants have resulted in FDA-approved treatments for rare diseases.

In 2015, NORD awarded a total of $230,000 in research grants for studies on Alveolar Capillary Dysplasia (ACD), Cat Eye Syndrome, Creutzfeldt-Jakob Disease (CJD), Cysathionine Beta-Synthase Deficiency, Lysosomal Storage Diseases, and Pseudomyxoma Peritonei (PMP).

Advancing Rare Disease Research

In December, the FDA awarded NORD, a grant as part of a cooperative agreement, to develop 20 natural history studies for specific rare diseases as part of a lottery system. A natural history study examines data on a disease over the course of its lifetime. NORD's registry systematically collects the data online, securely stores it, and enables study sponsors to analyze and share the outcomes with its patient communities and researchers.

Training the Community

In July of 2015, the University of Maryland and NORD were awarded the Eugene Washington Engagement Award from the Patient-Centered Outcomes Research Institute (PCORI). With this, we were able to empower patient organization representatives with the knowledge and skills to become more more engaged in patient-centered outcomes research for the treatment and cure of their disease. The training program was offered at NORD's 2015 Rare Diseases and Orphan Products Breakthrough Summit in October where 40 member organizations of NORD gathered for the exclusive training.

Making an Impact

$230,000 in research grants awarded

1,100+ Patients and Caregivers are actively using our new registry platform

Received $250k from FDA to support the development of 20 new natural history studies for rare diseases

NORD'S RARE SUMMIT:

In October, NORD gathered the entire rare disease community under one roof to discuss critical issue and identify new areas for collaboration and progress. The 2015 Summit was our most successful to date, with more than 500 attendees and speakers from leadership at the FDA and NIH, patients and patient groups, industry, researchers, investment, and academia.

RARE DISEASE DAY:

NORD was thrilled to once again serve as the U.S. host and sponsor of this international campaign, joining more than 85 countries in an international effort to raise the collective voice for rare diseases. 2015 was the biggest and most impactful year yet in the U.S. as we hosted 35 State House Events, trended on social media, and garnered billions of impressions in media coverage.

PORTRAITS OF COURAGE GALA:

In May of 2015, NORD hosted the Portraits of Courage gala, giving us the opportunity to honor the patients, caregivers, industry leaders, and those making an impact for the rare disease community at the National Building Museum in Washington, D.C. The black-tie optional event is NORD's largest annual fundraiser and a special evening to pause and celebrate our remarkable accomplishments as a community.

RUNNING FOR RARE:

Our inspiring charity running team, Running for Rare, participated in two marathons, Boston and Providence, and ran to Boston and Providence, raising $196,812 for NORD and its undiagnosed diseases program to help patients seeking to apply into the NIH program.

Together, Stronger. Our Membership:

(The) LAM Foundation
(The) Mastocytosis Society, Inc.
A Cure in Sight
A Twist of Fate-ATS
Acid Maltase Deficiency Association (AMDA)
Acromegaly Community, Inc
AKU Society of North America
Alagille Syndrome Alliance
Alpha-1 Foundation
Alport Syndome Foundation
Alternating Hemiplegia of Childhood Foundation
American Autoimmune & Related Diseases
American Behcet's Disease Association
American Brain Tumor Association
American Cleft Palate-Craniofacial Association
American Multiple Endocrine Neoplasia Support
American Partnership for Eosinophilic Disorders (APFED)
American Porphyria Foundation
American Syringomyelia & Chiari Alliance Project, Inc
Amyloidosis Support Groups, Inc.
APBD Research Foundation
Aplastic Anemia & MDS International Foundation, Inc
APS Type 1 Foundation
Association for Creatine Deficiencies
Association for Frontotemporal Degeneration (AFTD)
Association for Glycogen Storage Disease
Association of Gastrointestinal Motility Disorders, Inc (AGMD)
Ataxia Telangiectasia Children's Project, Inc (A-T)
Autoimmune Hepatitis Association
Autoinflammatory Alliance (3/25 formerly NOMID Alliance)
Basal Cell Carcinoma Nevus Syndrome Life Support Network
Batten Disease Support & Research Association
Benign Essential Blepharospasm Research Foundation, Inc
Bohring-Opitz Syndrome Foundation, Inc (BOS Foundation)
Breath of Hope, Inc
Bridge the Gap-SYNGAP Education & Research Foundation - 50
Cardio Facio Cutaneous International (CFC)
Castleman's Awareness & Research Effort (CARE) DBA Castleman Disease Collaborative Network
CCHS Network
Charcot-Marie Tooth Association
Children's Cardiomyopathy Foundation
Children's Craniofacial Association
Children's PKU Network
Children's Tumor Foundation, Inc
Cholangiocarcinoma Foundation
Chordoma Foundation - New 2016
Chromosome 18 Registry & Research Society
Chromosome Disorder Outreach, Inc
Chronic Granulomatous Disease Association, Inc
Cicatricial Alopecia Research Foundation (CARF)
Cloves Syndrome Community
Cluster Headache Support Group, Inc
CMTC-OVM -US
Congenital Hyperinsulinism International
Consortium of Multiple Sclerosis Centers
Cornelila de Lange Syndrome Foundation, Inc
Council for Bile Acid Deficiency Diseases
CureCADASIL/CADASIL Association, Inc
CURE HHT Foundation
Cure SMA
CurePSP
Curing Retinal Blindness Foundation
Cushing Support & Research Foundation, Inc.
Cutaneous Lymphoma Foundation
Cystinosis Foundation, Inc
Cystinosis Research Network, Inc
Daybreak Children's Rare Disease Fund
Desmoid Tumor Research Foundation
Dravet Syndrome Foundation
Dup15q Alliance
Dysautonomia Foundation Inc
ECD Global Alliance
Ehlers Danlos National Foundation
Erythromelalgia Association
Family Caregiver Alliance
Family Support Network of North Carolina
Fat Disorders Research Society, Inc.
Fibrolamellar Cancer Fdn
Fibromuscular Dysplasia Society of America
Fibrous Dysplasia Foundation
Foundation Fighting Blindness
Foundation for Ichthyosis & Related Skin Types, Inc
Foundation for Prader-Willi Syndrome
Friedreich's Ataxia Research Alliance (FARA)
Galactosemia Foundation
GBS/CIDP Foundation International
Genetic Alliance
Global Foundation for Peroxisomal Disorders
Glut1 Deficiency Foundation
Gut Check Foundation
Guthy Jackson Charitable Foundation

2015 NORD MEMBERS (continued)

Hemophilia Federation of America
Hereditary Disease Foundation, Inc
Hereditary Leiomyomatosis & Renal Cell Cancer Family Alliance (HLRCCFA) (part of VHL)
Hereditary Neuropathy Foundation
Hermansky-Pudlak Syndrome Network, Inc
Histiocytosis Association, Inc
Hope for Hypothalamic Hamartomas
Huntington's Disease Society of America
Hydrocephalus Association
Hypoparathyroidism Association, Inc.
Immune Deficiency Foundation
Incontinentia Pigmenti International Foundation
Indian Organization for Rare Diseases
International FOP Association, Inc.(Fibrodysplasia Ossification Progressive)
International Foundation for CDKL5 Research
International FPIES Association(International Association for Food Protein Entercolitis-name was changed)
International Myeloma Foundation
International Pemphigus & Pemphigoid Foundation (IPPF)
International Rett Syndrome Foundation(Cure Rett)
International WAGR Syndrome Association
International Waldenstrom's Macroglobulinemia Foundation
Intractable Childhood Epilepsy Alliance-ICE Epilepsy Alliance
Iron Disorders Institute
ISMRD
Jack McGovern Coats Disease
Jared's Juggernaut, Inc
Joshua Frase Foundation for Congential Myopathy Research
Julia's Wings Foundation, Inc
Kennedy's Disease Association, Inc
Klippel Trenaunay (KT) Support Group
LAL Solace, Inc
Les Turner ALS Foundation, Ltd
Liam's Land Organization, Inc
Life Raft Group
Lipoprotein a Foundation
Lowe Syndrome Association, Inc.
Lymphangiomatosis & Gorham's Disease Alliance, Inc
Marfan Foundation
Martin Mueller IV Achalasia Awareness Foundation, Inc.
M-CM Network
MEBO Research, Inc
Melorheostosis Association
Mesothelioma Applied Research Foundation
MitoAction
Moebius Syndrome Foundation
Morgan Leary Vaughan Fund, Inc.
Mowat-Wilson Syndrome Foundation
MPN Research Foundation
MSUD Family Support
Mucolipidosis Type IV Foundation, Inc. (ML4 Fdn)
Myasthenia Gravis Foundation of America, Inc
Myocarditis Foundation
Myositis Association
Myotonic Dystrophy Foundation
Narcolepsy Network, Inc.
National Adrenal Diseases Foundation
National Alopecia Areata Foundation
National Ataxia Foundation
National Brain Tumor Society
National Eosinophilia Myalgia Syndrome Network
National Foundation for Ectodermal Dysplasias
National Fragile X Foundation
National Hemophilia Foundation
National Lymphedema Network, Inc
National MPS Society
National Nieman-Pick Disease Foundation, Inc
National Organization for Albinism & Hypopigmentation
National PKU Alliance
National PKU News
National Spasmodic Dysphonia Association
National Spasmodic Torticollis Association
National Tay-Sachs & Allied Diseases Association
National Urea Cycle Disorders Foundation
NBIA Disorders Association
NephCure Kidney International
Neuroendocrine Tumor Research Foundation (formerly Caring for Carcinoid Foundation)
Neurofibromatosis Network
NICER Foundation
NTM Info & Research, Inc
Ocular Melanoma Foundation
Oley Foundation (The)
Organic Acidemia Association
OMSLife Foundation
Osteogenesis Imperfecta Foundation
Oxalosis & Hyperoxaluria Foundation
Pachyonychia Congentia Project
Pancreatic Cancer Action Network
Parent Project Muscular Dystrophy
Parkinson's Disease Foundation, Inc.
Phelan-McDermid Syndrome Foundation
Pitt Hopkins Research Foundation
PKD Foundation

Platelet Disorder Support Association

PMP Research Foundation

Prader-Willi Syndrome Association, USA

PRISMS (Parents & Researchers Interested in Smith-Magenis Syndrome)

PRP Alliance, Inc - prorated should be 50

PSC Partners Seeking A Cure (Primary Sclerosing Cholangitis)

Pulmonary Fibrosis Foundation

Pulmonary Hypertension Association

Rare & Undiagnosed Network

Rare Cancer Research Foundation

RASopathies Network USA

Recurrent Respiratory Papillomatosis Foundation

Reflex Sympathetic Dystrophy Syndrome Association

Rett Syndrome Research Trust (see note)

Rothmund-Thomson Syndrome Foundation

Sarcoid Networking Association

Sarcoma Foundation of America

SBS Cure Project

Scleroderma Foundation

Scleroderma Research Foundation

Short Bowel Syndrome Foundation

Shwachman-Diamond Syndrome Foundation

Snyder-Robinson Foundation, Inc.

Soft Bones, Inc.

Sotos Syndrome Support Association

Spastic Paraplegia Foundation

SSADH Association(Succinic Semialdehyde Dehydrogenase Deficiency)

Stevens Johnson Syndrome Foundation

Sturge-Weber Foundation

Target Cancer Foundation

Tarlov Cyst Disease Foundation

Tess Foundation

The JMML Foundation

TNA - The Facial Pain Association

Tourette Syndrome Association

Tuberous Sclerosis Alliance (National Tuberous)

Turner Syndrome Society of the United States

United Leukodystrophy Foundation

United Mitochondrial Disease Foundation

US Hereditary Angioedema Association

Vasculitis Foundation

Vestibular Disorders Association (VEDA)

VHL Alliance

Williams Syndrome Association

Wilson Disease Association

Wisconsin ME/CFS Association Inc (Myalgic Encephalomyelitis Chronic Fatigue Syndrome)

Worldwide Syringomyelia & Chiari Task Force Inc.

XLH Network, Inc.

Canadian PBC Society

Genetic Alliance Australia (formerly Assoc of Genetic)

Answering T.T.P. Foundation (Thrombotic Thrombocytopenic Purpura)

Cutis Laxa Internationale

EURORDIS

Canadian Organization for Rare Disorders - CORD

Genetic & Rare Disorders Organisation c/o Fighting Blindness

Proyecto Pide un Deseo Mexico, i.a.p

CMTC-OVM Cutis Marmorata Telangiectatica Congenita

Parent to Parent New Zealand, Inc.

Taiwan Foundation for Rare Disorders

CLIMB

Contact A Family

Syncope Trust & Reflex Anoxic Seizures (STARS)

Children with Spinal Muscular Atrophy, Ukraine-Kharkiv Charitable Foundation

Child Growth Foundation

HCU Network Australia

Wilhelm Foundation

Thank You, Corporate Council Members:

Business Member

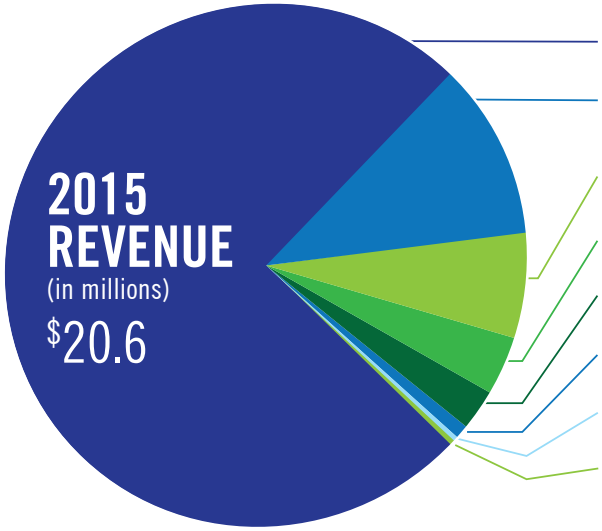
BDI Pharmaceuticals
Biotechnology Industry Organization (BIO)
Diplomat Specialty Pharmacy
Dohmen Life Science
Mallinckrodt
Invitae
Multicare Pharmaceuticals (AKA Expressa)
Walgreens Co.

Product Member

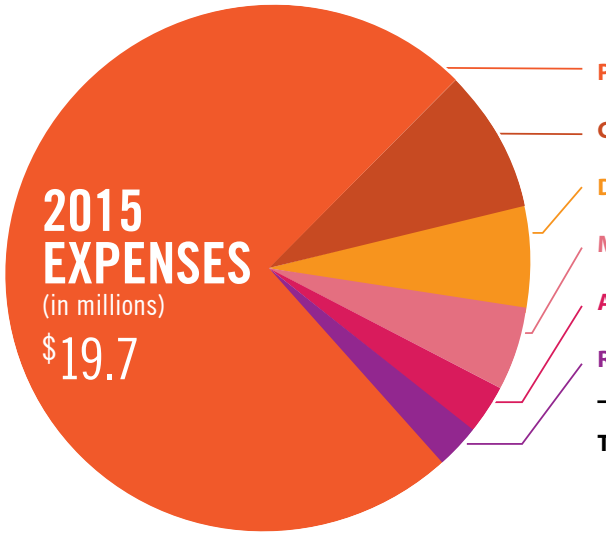
AbbVie Pharmaceuticals
Actelion Pharmaceuticals
Aegerion Pharmaceuticals, Inc.
Alexion Pharmaceuticals, Inc.
Allergan, Inc.
Baxter BioScience
Bayer Healthcare
BioGen Idec
BioMarin Pharmaceuticals, Inc.
Boehringer-Ingelheim Pharmaceuticals
Celgene Corporation
CSL Behring
Dyax Corporation
Genentech, Inc
Genzyme, a Sanofi Company
GlaxoSmithKline PLC
Hyperion Therapeutics, Inc.
Incyte Corporation
Ipsen Biopharmaceuticals, Inc
Jazz Pharmaceuticals PLC
Lundbeck, Inc.
Millennium Pharmaceuticals:
The Takeda Oncology Company
Novartis
Novo Nordisk
NPS Pharmaceuticals, Inc.
Onyx Pharmaceuticals, Inc.
Pfizer, Inc.
Raptor Pharmaceuticals Corporation
Recordati Rare Diseases
Retrophin, Inc
Shire Human Genetic Therapies, Inc.
Sigma Tau Pharmaceuticals, Inc.
SOBI Inc.
Therakos, Inc
Upsher-Smith Laboratories, Inc.
Vanda Pharmaceuticals
Vertex Pharmaceuticals Worldwide

Research Member

Achillion Pharmaceuticals
Agios
Alnylam Pharmaceuticals Inc
Amicus Therapeutics, Inc.
Asklepion Pharmaceuticals, LLC
Audentes Therapeutics
BioBlast Pharma
Catalyst Pharmaceutical Partners
Clementia Pharmaceuticals, Inc
Cortendo
Cytokinetics, Inc.
GlycoMimetics, Inc.
Insmed
Neuralstem Inc.
Neurotrope BioScience
Otsuka America Pharmaceutical, Inc.
REGENX Biosciences, LLC
Sarepta Therapeutics
Soligenix, Inc.
Spark
Stealth Peptides
Stemline Therapeutics, Inc.
Synageva BioPharama, Corp.
Vital Therapies, Inc
Xoma Corporation
Zafgen



Program Grants	$15,549,157	(75%)
Patient Assistance & Research Program Fees	$1,576,789	(8%)
Membership Dues	$1,254,505	(6%)
Special Events Revenue	$1,082,504	(5%)
Contributions & Bequests	$885,224	(4%)
Royalties & Other	$302,772	(1%)
Federated Grants	$23,817	(-1%)
Investment Income	$23,000	(-1%)
Total Revenue	**$20,697,768**	



Patient Services	$14,338,019	(72%)
General & Administrative	$1,880,855	(9%)
Development & Communications	$1,282,229	(6%)
Membership & Education	$987,419	(5%)
Advocacy	$663,907	(3%)
Research & Medical Scientific Affairs	$646,623	(3%)
Total Expenses	**$19,799,052**	

Note: The complete audited consolidated financial statements of the National Organization for Rare Disorders (NORD) for fiscal 2015 may be obtained by contacting NORD, 1900 Crown Colony Drive, Quincy, MA 02169, 617-249-7300.

2015 BOARD OF DIRECTORS

CHAIR
Sheldon M. Schuster, Ph. D.

SECRETARY
Josephine Grima, Ph.D.

TREASURER
Marshall L. Summar, M.D.

DIRECTORS

Frederick E. Barr, M.D., MSc

Ronald J. Bartek

Preston White Campbell, III, M.D.

Anthony Castaldo

Pat Furlong

Steven Grossman

Jonathan L. Haines, Ph.D.

Kim Hollander

Sarah Krug

Marybeth Krummenacker

Vicki McCarrell

Kevin McNaught, Ph.D.

Charles A. Mohan, Jr.

Frank J. Sasinowski

E. Michael Scott

SCIENTIFIC AND MEDICAL ADVISORY COMMITTEE

Marshall L. Summar, M.D., Chair
Division Chief, Genetics and Metabolism
Margaret O'Malley Chair of Molecular Genetics
Children's National Medical Center
Professor, Pediatrics, George Washington University School of Medicine and Health Sciences

Frederik Askari, M.D., Ph.D.
Associate Professor, Hepatology
Division of Gastroenterology
Director, Wilson Disease Program
University of Michigan Health System

Garrett E. Bergman, M.D.
Senior Director, Medical Affairs
Kedrion USA

Preston W. Campbell, III, M.D.
Executive Vice President for Medical Affairs
Cystic Fibrosis Foundation
Associate Professor of Pediatrics
Johns Hopkins Cystic Fibrosis Center

Robert M. Campbell, Jr., M.D.
Attending Physician
Division of Orthopedics
Director, Center for Thoracic Insufficiency Syndrome
Children's Hospital of Philadelphia

Harry (Hal) Dietz, M.D.
Victor A. McKusick Professor of Genetics and Medicine
Investigator, Howard Hughes Medical Institute
Johns Hopkins University School of Medicine
McKusick-Nathans Institute of Genetic Medicine

Gregory M. Enns, M.D.
Associate Professor of Pediatrics (Genetics)
Lucile Packard Children's Hospital
Associate Professor-Med Center Line, Pediatrics-Medical Genetics
Member, Child Health Research Institute
Stanford University School of Medicine
Marlene Haffner, M.D., MPH
President and CEO
Haffner Associates, LLC.

James E. Heubi, M.D.
Director, Clinical Translational Research Center
Co-Director, Center for Clinical and Translational Science and Training
Associate Dean, Clinical and Translational Research
Professor, Department of Pediatrics
Cincinnati Children's Hospital Medical Center

James F. Leckman, M.D.
Neison Harris Professor of Psychiatry
Psychiatry, Psychology and Pediatrics
Yale Child Study Center
Yale School of Medicine

Brendan Lee, M.D., Ph.D.
Robert and Janice McNair Endowed Chair in Molecular and Human Genetics
Professor of Molecular and Human Genetics
Professor of Molecular and Human Genetics
Baylor College of Medicine
Investigator, Howard Hughes Medical Institute

James E. Lock, M.D.
Cardiologist-in-Chief
Boston Children's Hospital
Alexandar S. Nadas Professor of Pediatrics
Harvard Medical School

Mary Jean Sawey, Ph.D.
Senior Vice President, Medical Director
ICC LOWE Inc.

Mendel Tuchman, M.D.
Scientific Director, & Mary Elizabeth McGehee Joyce Professor for Genetics Research
Children's Research Institute
Children's National Medical Center
Professor of Pediatrics, Biochemistry and Molecular Biology
George Washington University School of Medicine and Health Sciences

Susan Winter, M.D.
Medical Director, Genetics and Metabolism
Children's Hospital Central California

Doris T. Zallen, Ph.D.
Professor of Science and Technology Studies and Humanities
Virginia Tech University

NORD®
National Organization for Rare Disorders

rarediseases.org

Connecticut

55 Kenosia Avenue
Danbury, CT 06810
203-744-0100

Washington, D.C.

1779 Massachusetts Avenue, NW
Washington, D.C. 20036
202-588-5700

Massachusetts

1900 Crown Colony Drive
Quincy, MA 02169
617-249-7300

Rare Disease Day® is a registered trademark of EURORDIS.

NRD-1026